

205THD 62226660

Non-Transduced hMSCs in Immuno-competent mouse muscle

Day 3

Dil + hMSCs

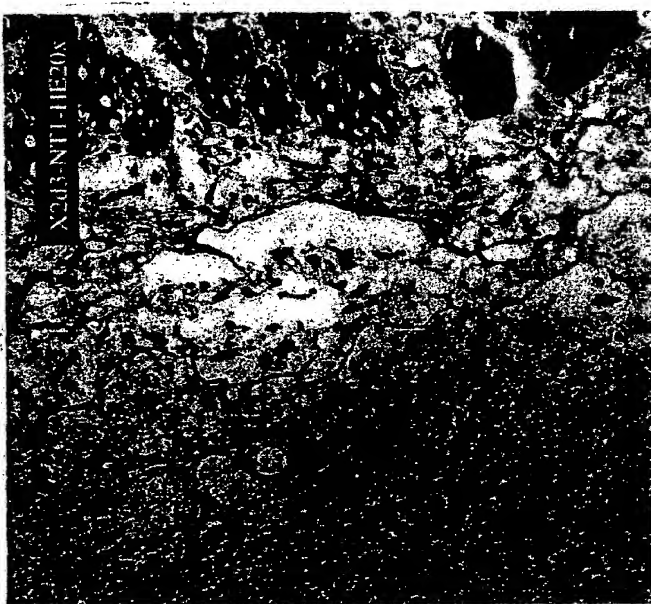
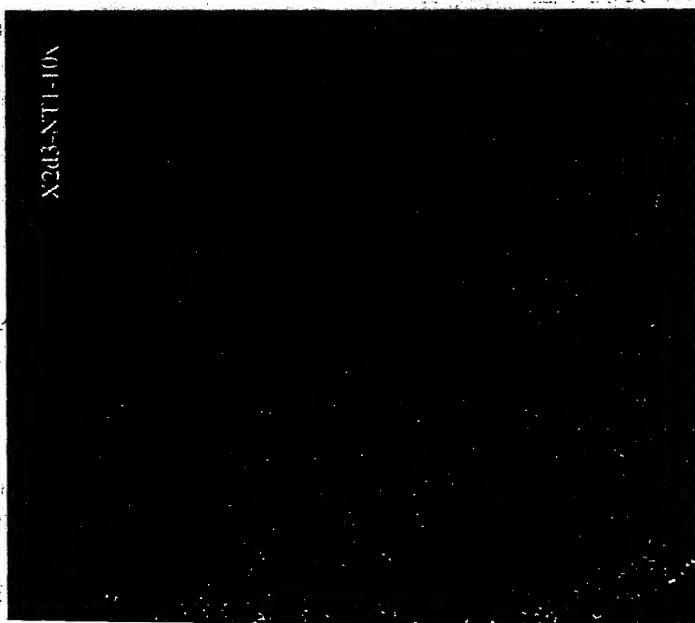


FIG. 1a

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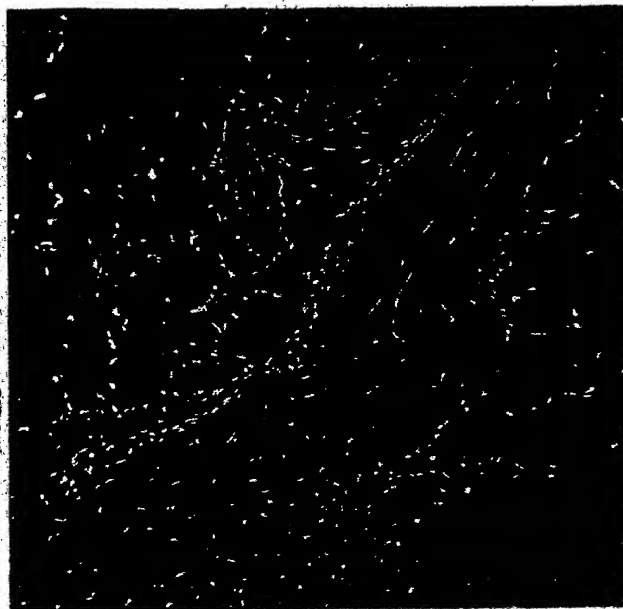
# FIG. 1b

Human MSCs in Mouse Muscle: Day 7

(H&E)



DiI labelled cells +  
Sybr-green staining  
of all nuclei



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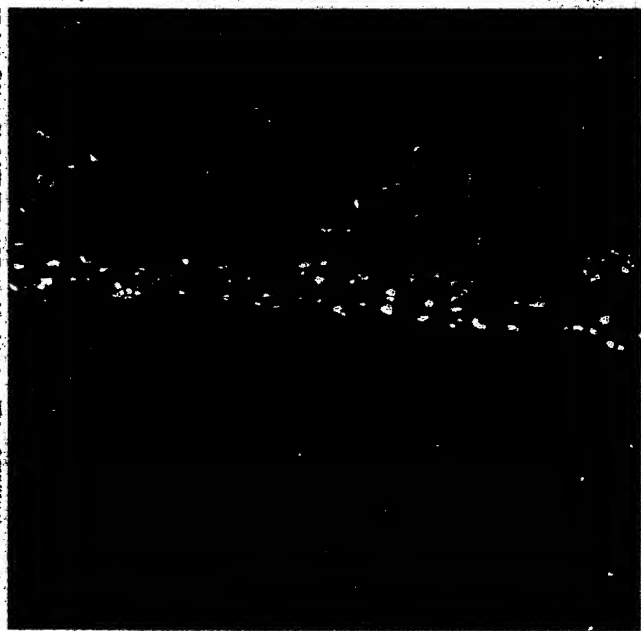
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+

# FIG. 1c

Human MSCs in Mouse Muscle: Day-7

(H&E, 20X)



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+

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FIG. 1d

Human MSCs in Mouse Muscle: Day 14

Fluorescent Field, 10X



H&E 40X



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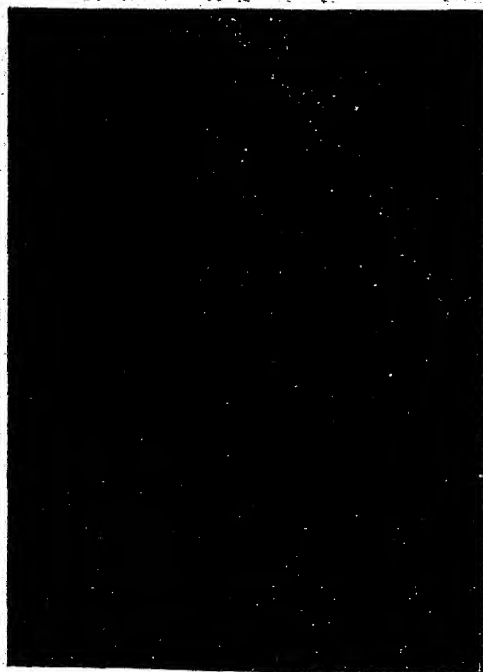
# FIG. 1e

DiI-Positive d-Gala Transduced hMSCs in  
Immuno-competent mouse muscle

d29



d43



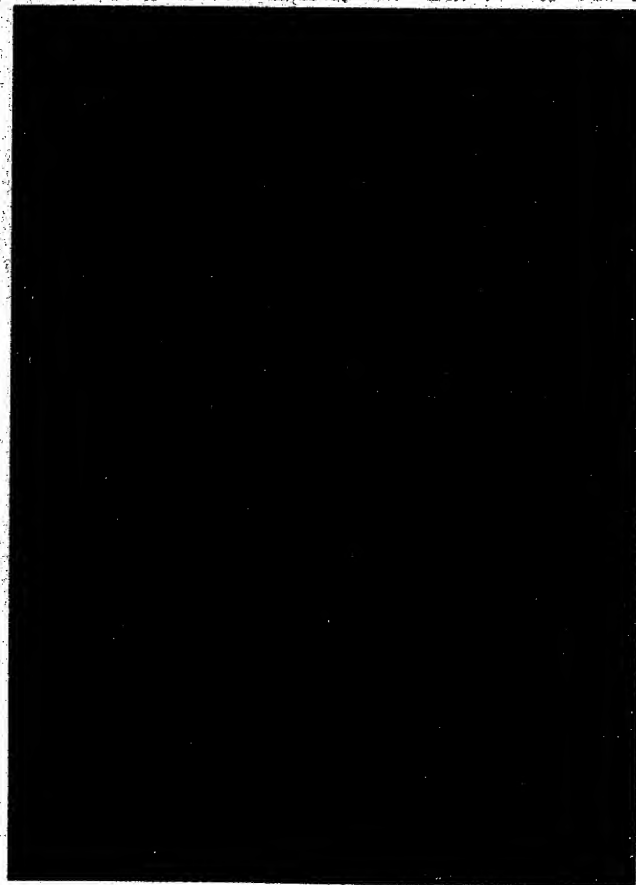
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## FIG. 1F

Human  $\alpha$  GalA-Transduced MSCs in Mouse Muscle: Day-57

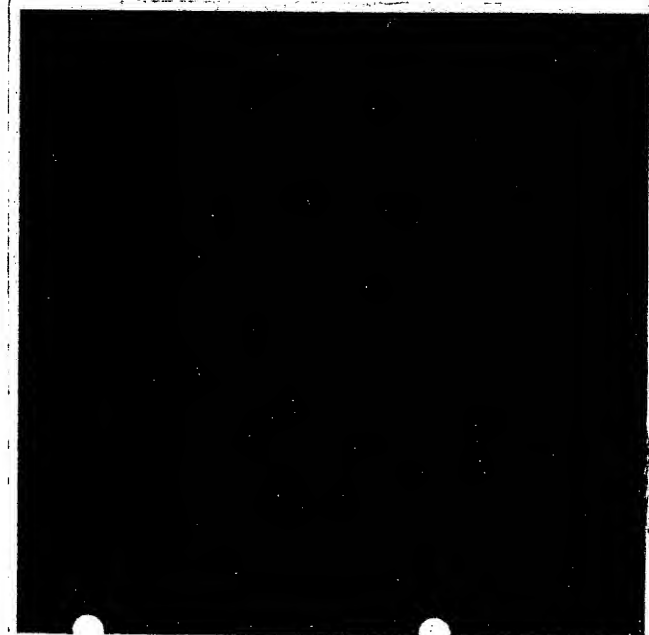
10X



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## FIG. 2

D3: IM injection of DiI-labeled PBMCs



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FIG. 3

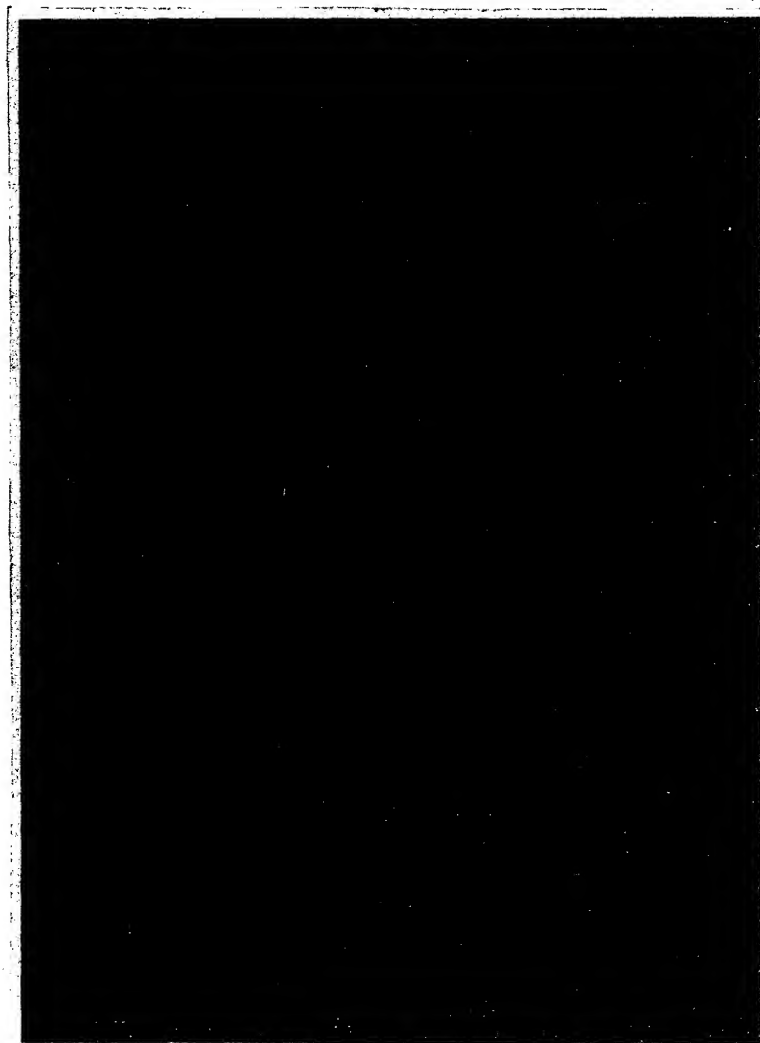
D3 IM injection of saline



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FIG. 4

Red and Blue Florescence Channels merged



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## FIG. 5

$\alpha$ GalA Immunostain of Muscle from Mouse  
injected IM with  $\alpha$ GalA-hMSCs: 14 days

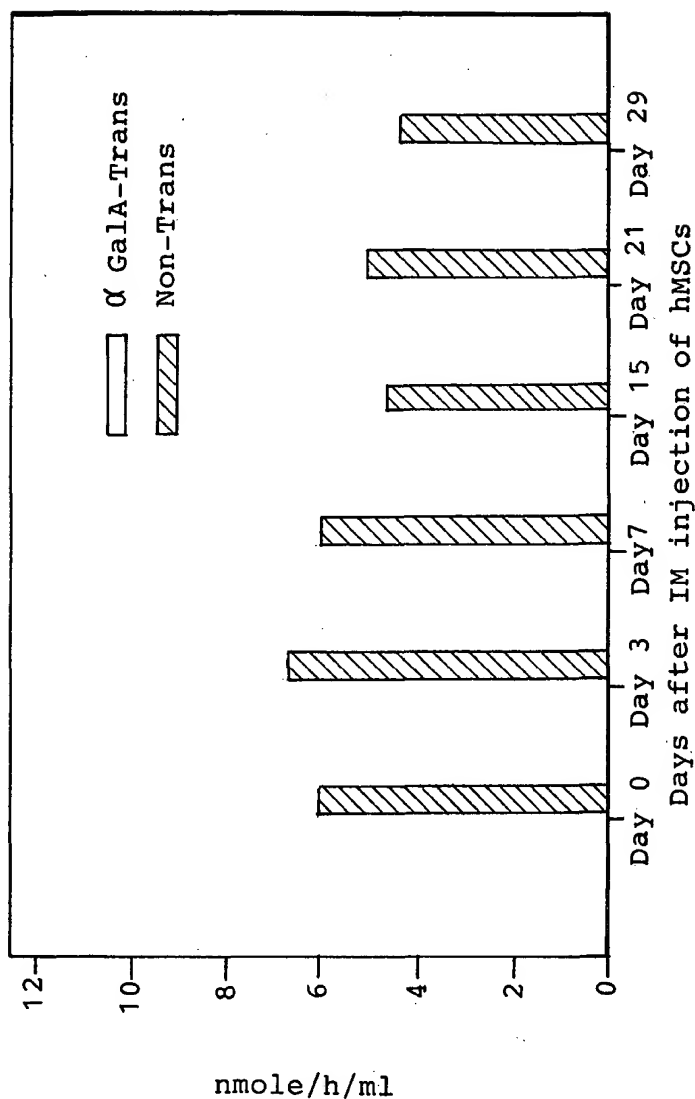


+

117 19

## FIG. 6

$\alpha$ -GAL activity in Plasma of Mice  
IM-injected with  $\alpha$ -GALA-Transduced h MSCs



#: No MSCs, \*n=2,

Fabry Mice plasma: 0.5 nmole/h/ml, PNAS 97:7515

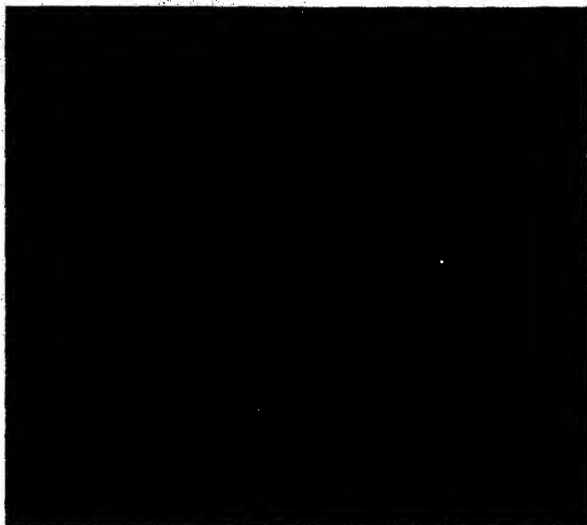
+

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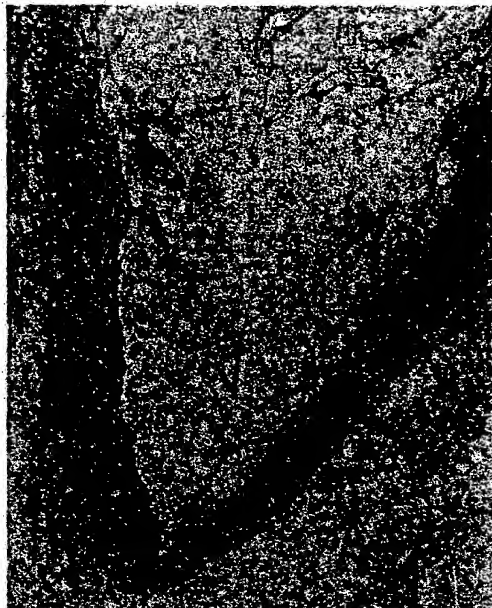
FIG. 7a

Human-specific Alu esquence staining of hMSCs  
injected into Immuno-competent mouse muscle

**Dil-positive cells**



**Alu-sequence positive cells**



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# FIG. 7b

Human MSCs in Mouse Muscle: 43 days

H & E



Dil Positive Cells

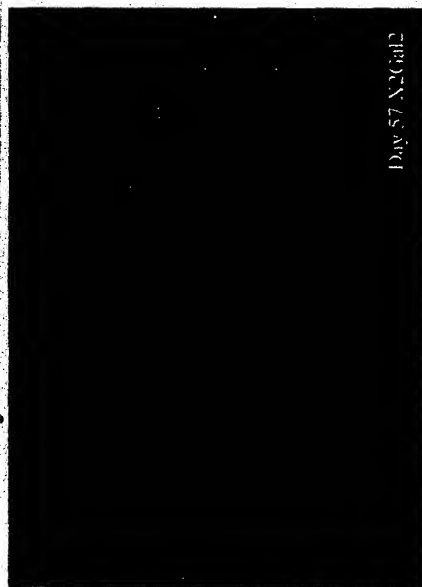


Alu-Positive cells



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Day 57 DiI/Alu



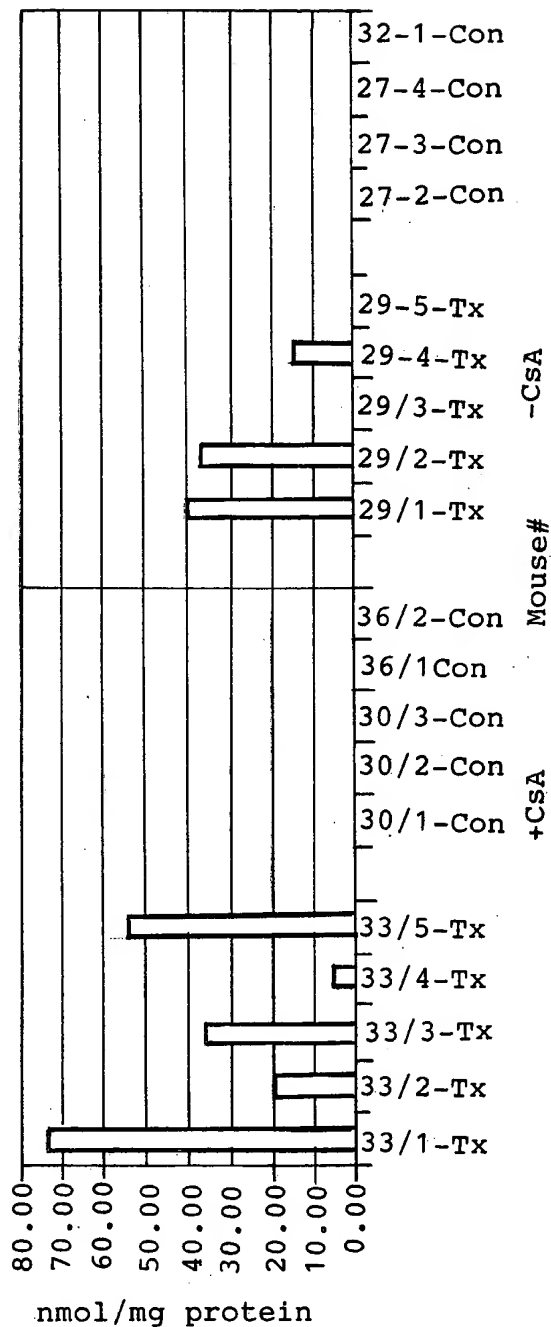
Day 57 NGal2



FIG. 7c

## FIG. 8a

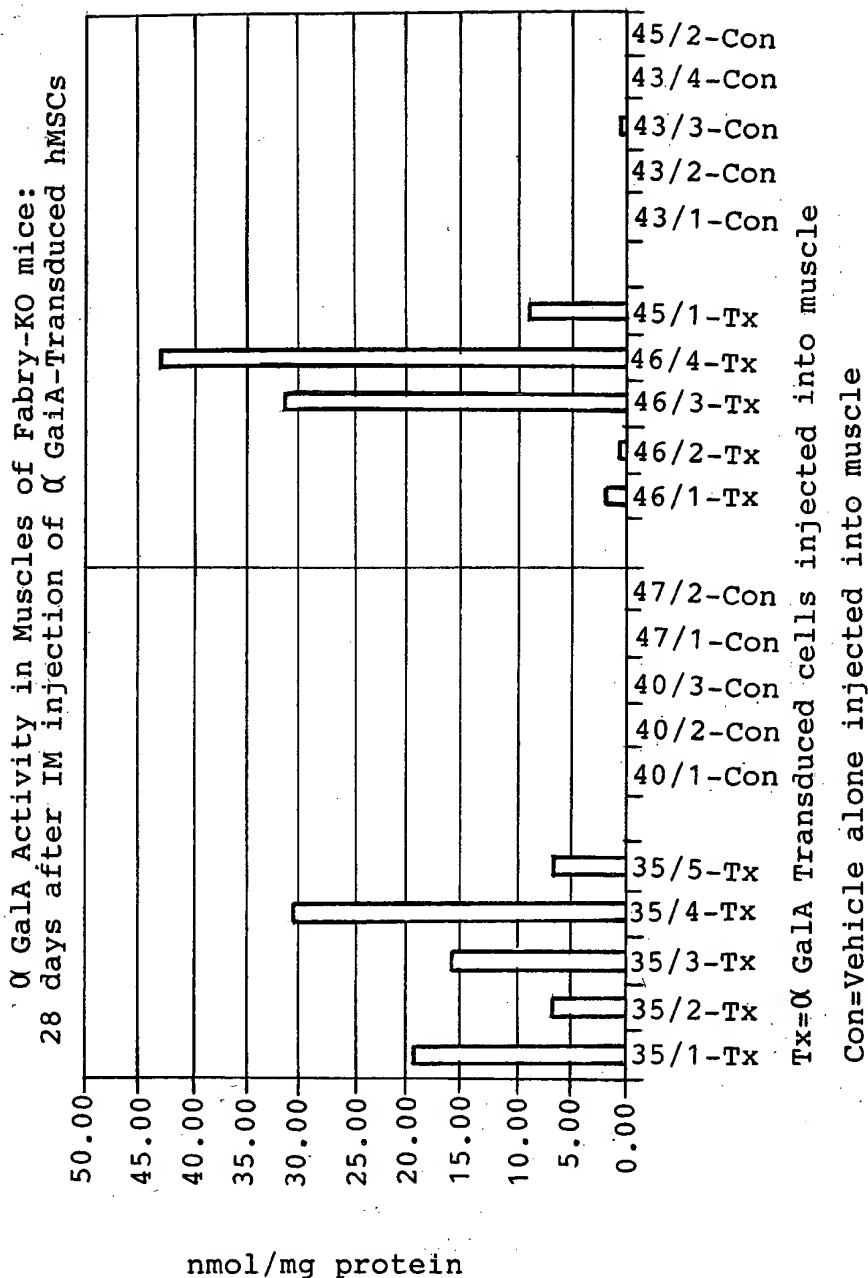
$\alpha$  Gala Activity in Muscles of Fabry-KO mice:  
14 days after IM injection of  $\alpha$  Gala-Transduced hMSCs



Tx =  $\alpha$  Gala Transduced cells injected into muscle

Con = Vehicle alone injected into muscle

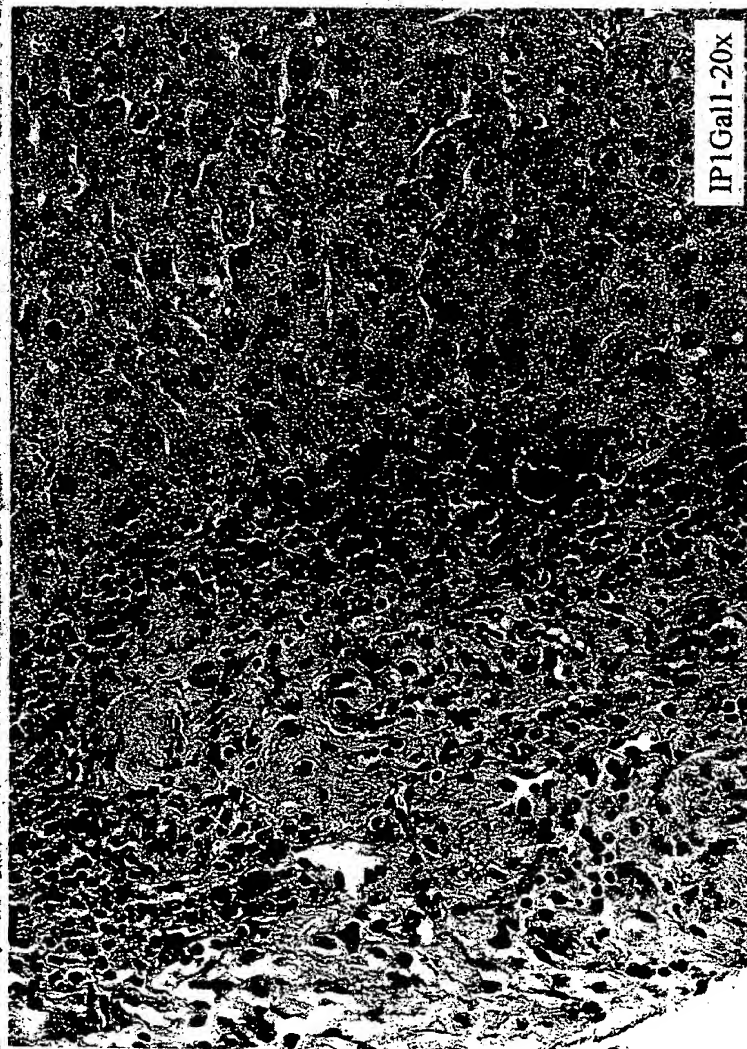
## F I G. 8b



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F1 G. 9a

αGala Immunostain of Liver from KO Mouse  
injected IP with αGala-hMSCs on Cultisphers:  
14 days

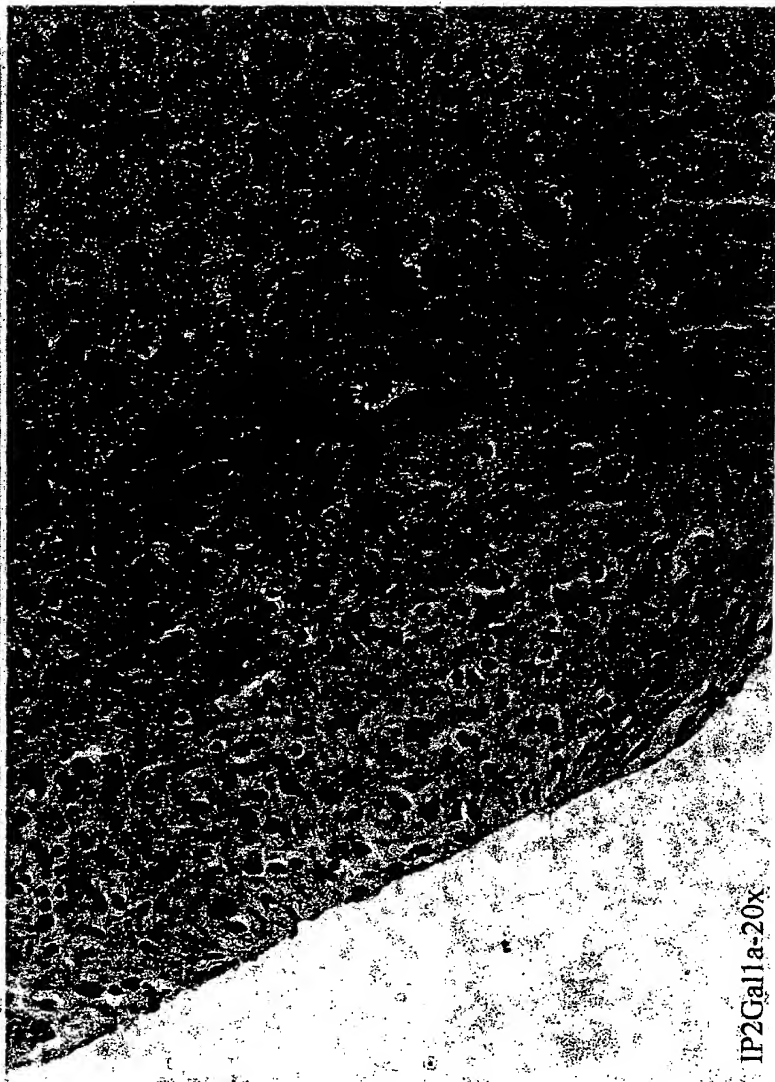


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## FIG. 9b

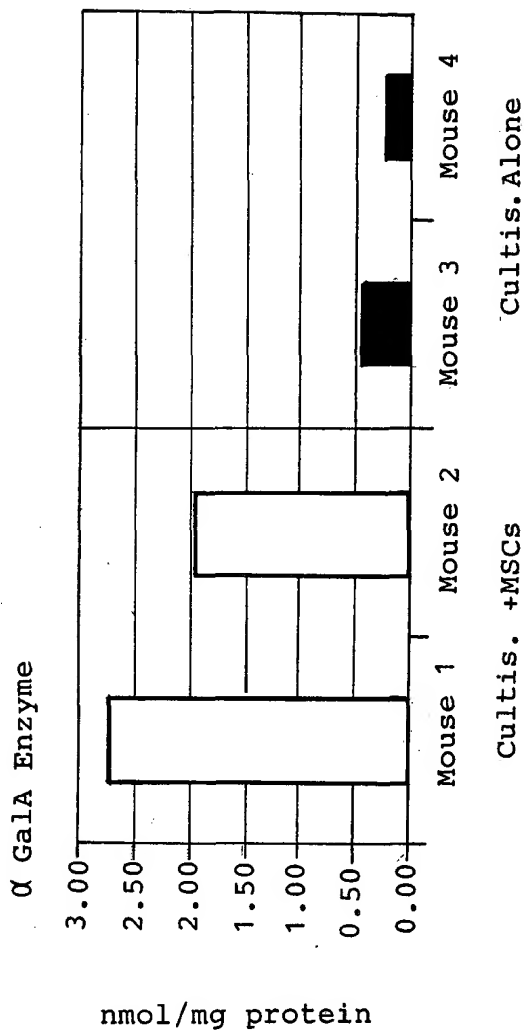
$\alpha$ GalA Immunostain of Liver from KO Mouse  
injected IP with  $\alpha$ GalA-hMSCs on Cultispheres:  
14 days



IP2Gal1a-20x

## FIG. 10

Intra-Peritoneal Delivery of  $\alpha$  Gala-Transduced hMSCs  
to Fabry Knock-Out Mice



Increase in  $\alpha$  Gala is 5% of normal mouse liver activity (41 nmole/mg)